Maryland
DEPARTMENT OF HEALTH

Vyondys 53 (golodirsen)

Vyondys 53 is an antisense oligonucleotide indicated for the treatment of Duchenne Muscular Dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.

I. Criteria for Initial Approval

Vyondys 53 will be considered for coverage when all of the criteria below are met, confirmed with supporting medical documentation.

- Treatment with Vyondys should be initiated, in male patients, before the age of 14 years.
- Prescribed by, or in consultation with a neurologist who specializes in treatment of Duchenne muscular dystrophy (DMD).
- Patients must have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.
- Patient has been on a stable dose of corticosteroids, unless contraindicated or intolerance, for at least 6 months.
- Documentation of the following pretreatment status:
    - Patient retains meaningful voluntary motor function.
    - Ambulatory status:
        - Ambulatory, with or without assistance devices, with 6 minute walk test (6MWT) of 180 m or greater (6MWT must be done within prior month).
    - Respiratory status:
        - Not ventilator dependent.
- Patient receiving ongoing physical therapy.
- Provider attestation of baseline and subsequent evaluation and monitoring as appropriate (e.g., hypersensitivity reactions and renal function).
- Patient is not receiving other RNA antisense therapy or gene therapy for DMD.

II. Criteria for Continuation of Therapy

All of the criteria for initial therapy (in **Section I.**) must be met; **AND** The provider must attest to a positive clinical response.

A positive clinical response is defined when the patient has responded to therapy compared to pretreatment baseline in one or more of the following categories:

- Stability, improvement, or slowed rate of decline in ambulatory function as demonstrated by the Six Minute Walk Test - 6MWT.
- Stability, improvement, or slowed rate of decline in respiratory function.
- Improvement in quality of life.

III. Dosing/Administration

Vyondys 53 must be administered according to the current FDA labeling guidelines for dosage and timing. The recommended dosing is as follows:

- 30 milligrams per kilogram, one time weekly.

IV. Length of Authorization for Therapy

Vyondys 53 will be authorized for 6 months when criteria for initial approval are met. Continuing therapy with Vyondys 53 will be authorized for 6 months.

V. Billing Code/Information

J1429 – Injection, golodirsen, 10 mg; 1 billable unit = 10 mg.

Prior authorization of benefits is not the practice of medicine nor the substitute for the independent medical judgment of a treating medical provider. The materials provided are a component used to assist in making coverage decisions and administering benefits. Prior authorization does not constitute a contract or guarantee regarding member eligibility or payment. Prior authorization criteria are established based on a collaborative effort using input from the current medical literature and based on evidence available at the time.

Approved by MDH Clinical Criteria Committee: 2/23/2021
Last Reviewed Date: 2/23/2021